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UNITED STATES DISTRICT COURT
NORTHERN DISTRICT OF CALIFORNIA
OAKLAND DIVISION

ASCENDIS PHARMA A/S, ASCENDIS PHARMA GROWTH DISORDERS A/S, and ASCENDIS PHARMA, INC.,

Plaintiffs,

v.

BIOMARIN PHARMACEUTICAL INC.,

Defendant.

Case No. 4:25-cv-03302-YGR

ASCENDIS'S REPLY IN SUPPORT OF ITS MOTION FOR A SPEEDY HEARING ON THE APPLICABILITY OF THE "SAFE HARBOR"

Date: July 15, 2025
[Motion for Shortened Hearing Time Pending; see D.I. 36]
Time: 2:00 PM
Courtroom: Courtroom 1, 4th Floor
Judge: Hon. Yvonne Gonzalez Rogers

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I. INTRODUCTION

This is precisely the type of case that Rule 57's speedy hearing procedure is made for. A short evidentiary hearing based on a limited and largely undisputed record can resolve the parties' discrete and dispositive dispute over the "safe harbor" provided by 35 U.S.C. § 271(e). Contrary to BioMarin's protestations, a speedy hearing will *not* require a complex mini-trial or lengthy pre-trial proceedings and *will* resolve the entire set of disputes between the parties in this Court and the ITC. Ascendis therefore respectfully requests that the Court order a speedy hearing that will resolve this case and save the Court, the ITC, and the parties tremendous expense and burden.

The evidence already in the record indicates that Ascendis has never sold TransCon CNP to anyone, anywhere, period. All of the TransCon CNP that has been made, whether imported or not, has been used for testing to obtain regulatory approval—all of which is activity protected by the safe harbor of 35 U.S.C. § 271(e). BioMarin speculates, based on the fact that Ascendis has filed a New Drug Application ("NDA") with the FDA, that Ascendis may have already entered into contracts for sales for importation of TransCon CNP for commercial purposes that would fall outside the safe harbor, but this speculation is simply mistaken. There are no such commercial contracts, as an evidentiary hearing will readily demonstrate. Accordingly, none of Ascendis's usage or importation of TransCon CNP, or contracts for the sale for importation of TransCon CNP, constitute an unfair import practice under Section 337 of the Tariff Act. These facts deprive the ITC of a statutory basis for maintaining its investigation.

Nevertheless, BioMarin filed an ITC complaint in derogation of Ascendis's safe-harbor rights, hoping that Ascendis would make some future act toward commercialization that would fall outside the safe harbor before the ITC investigation is complete, thus belatedly providing the missing statutory authority for its investigation. BioMarin's proposal that the ITC should adjudicate the safe-harbor defense along with all of the other complicated issues in the ITC investigation would vitiate the protections that Congress granted to companies like Ascendis to develop information to submit to the FDA without facing liability for patent infringement. Ascendis asks that this Court grant a speedy hearing on the narrow issue of its safe-harbor defense so that Ascendis can ask the ITC to end its baseless investigation. The parties may well have a

patent-infringement battle to fight someday. But not now.

The safe-harbor issue is narrow and the facts are straightforward. Although BioMarin tries to paint the issue as complex, the purported complexity consists of nothing but rank speculation on BioMarin's part. As the evidentiary hearing will expose, BioMarin has no evidence that Ascendis has entered into contracts to make TransCon CNP for commercial, rather than developmental or investigational use; BioMarin cannot deny that the bulk of the imported product is too old to be sold commercially consistent with the product's 24-month expiration date; BioMarin has not disputed and cannot dispute that every vial imported into the U.S. is labeled for investigational use only; and Ascendis has provided a declaration under penalty of perjury that any vials left over from clinical trials are destroyed rather than saved. Despite all this, BioMarin maintains that Ascendis might nevertheless somehow be stockpiling product in the U.S. for a future commercial launch. These allegations strain credulity.

Resolving the single, dispositive safe-harbor issue imposes only a limited burden on the Court and parties, one that is far less onerous and expensive than proceeding with full-blown patent litigation on the merits in the ITC or this Court. Ascendis is not seeking a preliminary injunction against BioMarin, let alone against the ITC. Ascendis is not asking this Court to rule on whether the ITC has an adequate statutory basis to maintain the investigation of BioMarin's complaint. Rather, Ascendis seeks a simple declaratory judgment that all of its allegedly infringing activity to date falls squarely within the safe harbor. Having this Court settle its safe-harbor rights will let Ascendis then seek termination of the ITC investigation and vindicate its Congressionally granted right to develop and test its drug without facing liability for patent infringement.

Although Ascendis has proposed a specific schedule as part of its motion, that schedule is, of course, merely exemplary. The Court should grant a speedy hearing on the safe-harbor defense as quickly as the Court deems practicable. Otherwise, as discussed below, Ascendis's safe-harbor rights will be lost.

II. ARGUMENT

A. The declaratory judgment Ascendis seeks would, if granted, suffice to resolve the current dispute between the parties.

As relevant to the present dispute, the ITC is authorized by statute to investigate three types of violation: "[1] [t]he importation into the United States, [2] the sale for importation, or [3] the sale within the United States after importation by the owner, importer, or consignee, of articles" that "infringe a valid and enforceable United States patent." 19 U.S.C. § 1337(a)(1)(B). BioMarin alleges that Ascendis has violated the Tariff Act by acts of [1] importation and [2] sale for importation. BioMarin then argues (at 7-8) that the declaratory judgment Ascendis seeks would not resolve the entire dispute between the parties because it would address only importation and not sale for importation. Not so. The allegations in the declaratory judgment complaint are not limited to questions of importation alone; they are broad enough to encompass sale for importation. By way of example, paragraph 34 of the Complaint alleges that "Ascendis's activities, *including* use and importation into the United States of the TransCon CNP investigational prodrug product, have solely been for clinical trials and the tests necessary for the submission of Ascendis's NDA to the FDA. The accused conduct of Ascendis is therefore not an act of infringement under the safe harbor of 35 U.S.C. § 271(e)(1)." D.I. 1 at 8 (emphasis added).

Regardless of whether one focuses on acts of importation or acts of sale for importation, there will be no violation of Section 337 if the act at issue falls within the safe harbor. BioMarin assumes that a present contract for a future commercial sale—i.e., a sale that, when it eventually happens, will not be covered by the safe harbor—could constitute a present violation of Section 337. To Ascendis's knowledge, no court has held that a contract for a future commercial sale counts as a present "sale for importation" under the Tariff Act. In any event, this Court need not reach that issue because there is no contract for future commercial sales of TransCon CNP, as an evidentiary hearing will confirm.

BioMarin has no contrary evidence; only speculation that Ascendis may have entered into such an agreement. The sole basis for this speculation is that Ascendis has submitted a New Drug Application ("NDA") to the FDA: BioMarin infers from the mere fact of that submission that it is

"more likely than not" that agreements for future sales of TransCon CNP after FDA approval are already in place. D.I. 41 at 8. BioMarin is simply wrong about this. *See, e.g.*, D.I. 32-1 (Toft Declaration) ¶ 4 ("Ascendis has not sold or offered to sell the TransCon CNP to later import into the United States.") Ascendis thus expects that the Court will find, based on an undisputed record, that there has been neither an importation nor a sale for importation falling outside the § 271(e) safe harbor—a finding sufficient to resolve the entire current dispute between the parties.

BioMarin incorrectly suggests (at 7, footnote 1) that its expected counterclaims for "any infringing conduct that falls outside Ascendis's § 271(e) safe harbor defense" would not be resolved by the requested declaratory judgment. But if the Court finds that there has been no activity outside the safe harbor, then BioMarin's patent-infringement counterclaim would fail due to a lack of any cognizable infringing act. In the unlikely event that the Court were to find that there has been some activity outside of the safe harbor, then the ITC case would continue. But in that circumstance, Ascendis would invoke the mandatory stay of BioMarin's patent-infringement counterclaim under 28 U.S.C. § 1659 to avoid burdening the parties and the Court with duplicative parallel litigation.

B. The facts central to the safe-harbor defense are not complex and are largely undisputed.

BioMarin does not dispute that TransCon CNP has not yet been approved by the FDA or any other regulatory authority and has never been sold commercially. The Toft Declaration submitted in support of the present motion for a speedy hearing states affirmatively that "[a]ll of the TransCon CNP that has ever entered into the United States, in any form, is used exclusively to obtain clinical and other testing data that will be provided to the FDA for regulatory approval." D.I. 32-1 ¶ 4. Moreover, all of the vials of TransCon CNP drug product imported into the U.S. are labeled for investigational use only. *Id.* ¶ 5. BioMarin has not disputed this or even suggested that it could dispute these unremarkable, incontrovertible facts.

Nevertheless, in an effort to make the factual issues appear more complicated than they are, BioMarin pretends that it will be necessary to trace every batch and every vial of TransCon CNP that has ever been made. The nub of BioMarin's argument is that if any excess amounts of

product have been imported, they hypothetically could be stockpiled for future commercial sale that would fall outside the safe harbor. *Cf. Amgen, Inc. v. Hospira, Inc.*, 944 F.3d 1327, 1340 (Fed. Cir. 2019) (affirming verdict based on some drug product falling outside the safe harbor where documentary evidence showed that certain batches were mostly "commercial inventory"). But as the Toft Declaration makes plain, "Ascendis does not stockpile TransCon CNP in the United States." D.I. 32-1 ¶ 6. BioMarin's speculation otherwise is not evidence, and is no reason to avoid a speedy hearing that can dispose of this case.

As Ascendis has already explained and as an evidentiary hearing will quickly confirm, BioMarin's theory (at 9-10) that 24 kg of drug substance imported in the 2020-2021 timeframe represents more than is needed for regulatory purposes rests on a misreading of the relevant import records. *See* D.I. 32 (Motion) at 4-5. In fact, about 94% of that 24-kg amount is water and so does not represent more drug substance than would be needed for FDA-approval-related purposes. D.I. 32-1 (Toft Decl.) ¶ 7. Furthermore, Ascendis is seeking FDA approval to market TransCon CNP with a 24-month shelf life. *Id.* ¶ 11. There is therefore no need to track down vials of TransCon CNP drug product made more than 24 months ago, since that product would not be eligible for commercial sale in the U.S., even if any of it had been stockpiled, which is not the case.

Regarding the more recent imports, Ascendis already has submitted evidence that all of the vials of drug product have been used for clinical trials in the U.S. *Id.* ¶ 6. All of them are labeled for investigational use only. *Id.* ¶ 5. Any vials left over from the clinical trials are accounted for and destroyed. *Id.* ¶ 6. BioMarin's suggestion (at 10) that it needs discovery into the ultimate fate of every single vial ever made not only ignores the issue of product expiration dates but also implies, without any factual basis, that Ascendis might be secretly removing the "for-investigational-use-only" labels from the handful of vials left over from clinical trials to serve as the nucleus of a commercial launch if and when the FDA approves its product. Such wild speculation does not justify the excessively burdensome and granular discovery that BioMarin purports to need nor its attempt to avoid a speedy hearing.

Nor is there any need for discovery from non-parties, since Ascendis is the importer of record for all imports of TransCon CNP, which is the only article accused of infringing BioMarin's

patent. BioMarin claims to want to take discovery from companies that supply reagents to Ascendis—for example, NOF Corporation in Japan. BioMarin asserts (at 10) that obtaining discovery from Japan via letters rogatory can take a long time. But BioMarin never says why such discovery is relevant, and it is not. BioMarin does not allege that NOF has ever imported TransCon CNP (or anything else, for that matter) into the U.S., or that NOF has sold TransCon CNP for importation. NOF makes mPEG-maleimide, an ingredient that is used to make TransCon CNP in Europe. D.I. 32-1, ¶¶ 8, 9. That activity simply has no bearing on whether Ascendis's acts do or do not fall within the § 271(e) safe harbor. BioMarin's professed desire for discovery of non-parties in exotic locations is a mere distraction.

Similarly unpersuasive is BioMarin's observation that discovery has been underway in the ITC for some 35 days and is not yet complete. There are many issues in dispute in the ITC proceedings, including infringement, invalidity, the economic and technical prongs of BioMarin's domestic industry, and public-interest factors. So it is not surprising that discovery on the whole case is taking more than a month. But little if any of that discovery bears on the question of whether Ascendis has or has not been undertaking activities outside the § 271(e) safe harbor.

C. Ascendis's rights under the § 271(e) safe harbor will be lost in the absence of a speedy hearing.

Since this motion was filed, the FDA has accepted Ascendis's NDA for filing and has granted it priority review, meaning that the FDA will undertake to complete its initial review of the application in six months rather than the usual ten months. Priority review is reserved for "applications for drugs that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions." *See* "Priority Review," FDA, https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review (visited Jun. 14, 2025). The FDA's grant of priority review is thus further evidence that TransCon CNP, with its once-a-week dosing schedule, if approved, would be a "significant improvement" over BioMarin's existing Voxzogo® product, which requires that children receive daily injections.

The FDA target date for completion of initial review is November 30. At that time, the

FDA could potentially approve the application, although frequently the approval process takes years even for drugs granted priority review. By opposing the present motion for a speedy hearing, BioMarin is plainly hoping to run out the clock on Ascendis's statutorily provided safe-harbor defense. That is, if the FDA were to grant approval while the ITC investigation was still ongoing, Ascendis might well make preparations to launch its product commercially and so take some action with regard to TransCon CNP that falls outside the safe harbor, thus—however belatedly—providing a statutory basis for the ITC to continue its investigation. That is, BioMarin hopes that, if it can delay resolution of the safe-harbor issue long enough, then the defense could cease to apply to at least some of Ascendis's acts.

But the reason Congress created the safe harbor is to avoid precisely what BioMarin is doing here: imposing the significant costs and burdens of patent-infringement litigation on a drug company for acts of alleged infringement that are necessarily part of obtaining FDA approval to bring competitive new medicines to patients who need them quickly. The right to be free of such harassment is one that Congress has granted by statute to companies like Ascendis, and BioMarin is simply hoping that if it can drag out proceedings in the ITC and in this Court long enough, the ITC will allow it to continue an investigation that, as of now, has no legitimate basis.

Furthermore, there is a significant chance that the FDA will *not* grant final approval to Ascendis's TransCon CNP NDA before the evidentiary record in the ITC has closed. If that happens, then the ITC will adjudicate the safe-harbor issue along with all of the other many issues in the investigation, and the parties will have spent, collectively, tens of millions of dollars only to find out that the ITC lacked a statutory basis for the investigation because all of the putative violations of the Tariff Act are covered by the § 271(e) safe harbor. And then, presumably, once the FDA grants approval, BioMarin will file a new ITC complaint, and the whole process will start all over again. This Court can avoid such a senseless waste of party and public resources. One object of the current declaratory judgment action is to establish that there is no present statutory basis for the ITC to maintain its investigation so that the ITC will terminate it *before* the parties have wasted tens of millions of dollars on it.

If the ITC had elected to adjudicate the safe-harbor defense on an expedited basis, then the

present declaratory judgment action would not be necessary. BioMarin makes much of the fact that Ascendis waited several weeks before filing the present speedy hearing motion. Ascendis waited not only to avoid what it anticipated would be complaints from BioMarin about briefing the motion before BioMarin had even responded to the complaint but also to give the ITC a chance to agree to expedited consideration to the safe-harbor defense, which would have mooted the present action.

If, after a speedy hearing on this limited issue, the Court determines that Ascendis's conduct to date falls squarely within the safe harbor, then Ascendis can take that finding to the ITC and seek an end to the current wasteful investigation. But if the Court declines to grant a speedy hearing on the narrow safe-harbor issue, then Ascendis's statutory safe-harbor rights would be rendered a nullity. Either the FDA will grant approval before the ITC's evidentiary record is closed, in which case BioMarin's gamesmanship in filing a complaint before any even arguable violation has occurred will be rewarded, or else the FDA will not grant approval before the ITC's evidentiary record is closed, in which case Ascendis will have been dragged through an expensive and disruptive ITC investigation for nothing. In either case, the Congressionally mandated benefit of the safe harbor to develop and seek approval for its drug before having to deal with allegations of patent infringement will have been lost.

III. CONCLUSION

Ascendis's motion for speedy hearing should be granted.

Dated: June 17, 2025

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